Testimony of Mary Nathan

before the

U.S. House of Representatives

Committee on Oversight and Government Reform

"Access to Life-saving Medicines Act of 2007"

Monday, March 26, 2007

Mr. Chairman and distinguished members of the Committee, I want to thank you for the opportunity to testify before you today. My name is Mary Nathan and I am affected by Gaucher Disease.

As one of the 4,800 people being treated worldwide with Cerezyme®, I understand, in a very practical way what it means to be alive because of a recombinant biologic medicine. I also understand what happens when the cost of a life-saving drug is unaffordable.

What is Gaucher Disease?

Gaucher disease is a rare genetic disorder characterized by the deficiency of an enzyme necessary to break down fats called glycolipids. Because the enzyme is in short supply, lipids collect in the spleen, liver, bone marrow, and other organs. Left uncheck the accumulation of lipids causes problems such as anemia and bleeding, organ dysfunction and abdominal enlargement, deterioration of the joints and bones, breathing problems, fatigue, and a reduced ability to fight common infections.

Gaucher disease is classified into three categories - Types I, II and III. Type I, the adult form, is usually the least severe and is also the most common. Occurring among all racial and ethnic groups, this condition affects an estimated 20,000 Americans. It strikes one in 40,000 of the general population, and one in 600 Jews of Eastern European heritage.

The disease differs significantly from person to person. Some are asymptomatic or have minimal symptoms, while others experience severe and chronic problems causing life-long disability.

My Journey with Gaucher Disease

Gaucher disease has been my constant companion since my diagnosis in 1966 at the age of eleven. Very little was known about the disease at the time, and treating physicians mostly had to guess what was happening to me and what course of treatment to pursue.

Given the increased size of my spleen and my low blood count, doctors scheduled me for a splenectomy within weeks of my diagnosis. I bounced back from the operation. In fact, I was doing so well that I was able to attend an international children's camp in Denmark.

That wonderful adventure came to an abrupt end when I was hospitalized in Denmark with a high fever, excruciating pain and an inability to walk. I was immediately flown home in a stretcher and met in New York by my worried parents. We were to learn later that lipids had migrated quickly to my bones since doctors had removed the one place where they are most likely to collect – the spleen. We also learned that I had experienced a Gaucher bone crisis, a painful episode that would be repeated often as the disease progressed.

By the time I entered college, there was little doubt that I had a severe form of Type I Gaucher Disease. My blood count remained low and I had difficulty walking due to a curvature of my leg bone and a deteriorating hip joint. Through all the pain and discomfort, I eventually graduated, found a job and got my own health insurance.

At the age of 23, I underwent orthopedic surgery to straighten my leg and replace my destroyed hip joint. After a long recovery, I was able to walk without pain for the first time in many years. In better health, I started graduate school and focused on my career.

This respite lasted until 1988 when the prosthesis implanted when I was 23 became painful and unstable. Again, I underwent surgery. Fortunately, I was able to arrange time off from my demanding job as executive director of a professional association.

Unlike previous surgeries, this time I began to experience complications that left me fighting for my life. The Gaucher-related problems escalated rapidly, requiring hospitalization for the better part of a year. My red blood cell count was dangerously low, depriving my bones of oxygen. I then began to experience an ongoing cascade of bone infarcts, vertebrae fractures, and a serious fracture of my other hip.

To head off further damage, my surgeon decided on a simple surgery to repair my other hip. Known as a girdlestone procedure, it is a surgery of last resort. Few patients ever walk again.

Still seriously ill and confined to a wheelchair, I was discharged from the hospital. Physically and mentally exhausted and terrified of living in a wheelchair, I was financially ruined and out of a job. I now faced a life completely dependent on family and friends, not knowing what to expect or how to cope with the devastation of my disease.

Breakthrough Therapy Discovered

I turned to family and friends for help and within a few months, I was living in Massachusetts with a friend. This arrangement worked well because I could consult with the Gaucher Disease specialists at Massachusetts General Hospital. They provided the first glimmer of hope by suggesting that my hip problems might be corrected if my blood picture and stamina improved.

What happened next marked an historic medical discovery that would change the course of my disease. After 30 years of intense research, scientists at the National Institutes of Health developed a treatment for Gaucher Disease, and in April 1991, the Food and Drug Administration approved a commercial version Ceredase®. The enzyme my body could not produce could now be found in a vial.

Treatment with Ceredase® began shortly after approval. I saw a dramatic improvement during the first year. As treatment continued, my blood count returned to normal and the size of my liver decreased. Even the level of pain seemed better and the bone flare-ups vanished.

After three years of enzyme replacement therapy, my overall health improved to the point where reconstructive hip surgery was possible. The remarkable surgery that gave me a new right hip was complicated and painful, but I did make slow, steady progress. On my fortieth birthday in November 1994, after seven years in a wheelchair, I took my first real steps. As the months passed, I was able to walk further distances and before long I could walk unaided.

Cost of Care

There is no question in my mind that I am alive today because of the development and commercialization of the orphan drug Ceredase®. Others with Gaucher Disease have similar stories and, like me, are grateful to the Genzyme Corporation for bringing Ceredase® to market.

What concerns many of us, however, is that the miracle drug is priced out of the reach of individuals and thus poses unprecedented challenges for patients who need the drug, the doctors who treat us, for employers struggling with the high cost of health insurance, and for insurers and government programs helping to pay our medical bills.

The Genzyme Corporation sold its flagship product Ceredase® from 1991 to 1994 when most patients were converted to Cerezyme®, the company's newly approved orphan drug made in genetically engineered cells rather than from purified human placentas.

The cost of Cerezyme differs from patient to patient because dosages are based on body weight. My dosing regimen is 60 units per kilogram of body weight per infusion. At 130 pounds, my treatment runs about $12,643 per administration or about $303,432 a year for 24 doses.

In my case, the cost of administering the drug is another $10,000 a year, while related expenses for testing and monitoring my response to the drug and overall health are $15,000 annually.

This brings the cost for all charges related to my treatment to $328,432 a year. Over a sixteen year period, I estimate that the payments for my drug have reached well over $4.5 million.

Health Insurance Issues

Gaucher patients struggle to get and maintain sufficient health insurance to cover their medical bills. This task is made even more difficult because of business policies and common trends found throughout the health insurance industry. Pre-existing disease can present formidable barriers to those seeking insurance. Some insurance companies permanently exclude specific conditions while others set a waiting period of several months to a year. Many patients, including myself, purchase an individual policy while covered by other insurance. This allows full coverage until the waiting period ends.

The insurance practice of placing a dollar limit or "cap" on lifetime benefits is a cause of great anxiety among Gaucher patients. While many patients and their families have good coverage, others have already exceeded their million dollar limits. In these instances, patients try to purchase a new policy and are often assisted by reimbursement specialists at the Genzyme Corporation.

Conclusion

Mr. Waxman, I want to thank you for introducing the "Access to Life-Saving Medicines Act." It is time to make lifesaving biotech therapies accessible and affordable to the millions who need them. There is no reason why biogenerics can not take their rightful place in America's marketplace along side generic drugs.

The wave of the future in medicine is biotechnology-derived products and devices to treat rare diseases like mine, and those diseases affecting wider populations. But the question is, "Can the healthcare system withstand the costs associated with these miracle drugs?" I personally do not feel that it can.

I read recently that your legislation could initially save the American people about 10 percent to 20 percent over existing biotech therapies. For me, that would be a savings of between $30,343 and $60,686 annually. If you do the math for the 4,800 patients currently on Cerezyme®, just based on the cost of my treatments and my body weight, the healthcare system could save between $145 million and $291 million annually.

Mr. Chairman, patients like me need and want safe and effective medicines that they can afford.

Today there are over 300 orphan drugs treating well over 14 million people across the United States. Twenty-one percent of those are biologics. But there are millions more waiting on the sidelines hoping against hope that one day they, too, will have a drug or biologic to treat their disease. The "Access to Life-Saving Medicines Act" will create competition in the marketplace and, in turn, foster innovation.

A balance must be struck that encourages innovation, yet allows more affordable follow-on biologics to come to the marketplace. Hopefully, in years to come, there will be many more than 300 orphan products available to patients.

Thank you.